Neurocrine Biosciences Presents New Two-Year CRENESSITY® (crinecerfont) Data Demonstrating Durable Hormone Control, Reduced Glucocorticoid Exposure and Meaningful Clinical Improvements in Pediatric Patients with Classic Congenital Adrenal Hyperplasia

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- At two years, 60% of patients who were overweight or obese at baseline experienced clinically meaningful improvements in body mass index, and 61% of those with insulin resistance at baseline were no longer insulin resistant
- Improved outcomes associated with excess androgens, including acne and androstenedione-to-testosterone ratio were also observed
- CRENESSITY delivered sustained reductions in adrenocorticotropic hormone and 17-hydroxyprogesterone, while enabling lower, more physiologic glucocorticoid dosing

SAN DIEGO, May 1, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the presentation of new two-year data from the Phase 3 CAHtalyst® Pediatric study demonstrating durable androgen control, sustained decreases in glucocorticoid (GC) doses and meaningful improvements in clinical outcomes associated with excess androgens and long-term GC exposure in children and adolescents with classic congenital adrenal hyperplasia treated with CRENESSITY® (crinecerfont).

Consistent with these findings, patients with classic congenital adrenal hyperplasia (CAH) continued to experience substantial reductions in adrenocorticotropic hormone (ACTH) and 17-hydroxyprogesterone (17-OHP) while achieving lower, more physiologic GC doses. These data build upon previously reported one-year clinical outcomes results and were presented at the Pediatric Endocrine Society 2026 Annual Meeting in San Francisco.

"These two-year findings showed that CRENESSITY achieved durable reductions in both androgen levels and glucocorticoid doses in children and adolescents with classic congenital adrenal hyperplasia, a population particularly vulnerable to the long-term health impact of excess hormone exposure during growth and development," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "Improved hormonal control was associated with meaningful improvements in clinical outcomes, including body mass index and insulin resistance, supporting healthier outcomes as patients transition into adulthood."

These findings highlight the broader clinical implications of improved hormonal control and reduced GC exposure in pediatric patients with classic CAH.

The analysis included 86 participants aged four to 17 years who completed up to two years of CRENESSITY treatment in the open-label extension of the CAHtalyst Pediatric study. Mean changes from baseline in key hormonal markers, including ACTH and 17-OHP, measured prior to the morning GC dose, were reported through Month 24.

ACTH and 17-OHP Reductions
Meaningful and durable reductions in mean ACTH and 17-OHP were observed at 12 months, with further reductions observed at 24 months, despite sustained decreases in GC doses during the same period.

Measure	Baseline	Change from Baseline at Month 12	Change from Baseline at Month 24
Mean ACTH (pg/mL)	329 (n=103)	-118 (n=93)	-157 (n=82)
Mean 17-OHP (ng/dL)	8,682 (n=103)	-1,698 (n=94)	-1,924 (n=84)
Mean daily GC dose (mg/m²/day hydrocortisone equivalents), observed	16.4 (n=103)	-2.9 (n=94)	-3.2 (n=84)

Clinically meaningful improvements were observed across outcomes related to hormone control, excess androgens and long-term supraphysiologic GC exposure among relevant participants at baseline.

"In pediatric patients with classic congenital adrenal hyperplasia, excess androgens can accelerate bone age and drive early puberty, which can result in reduced final adult height. This, along with chronic supraphysiologic glucocorticoid exposure, can impact cardiometabolic health and quality of life in patients with CAH," said Mimi Kim, M.D., MSc, Associate Professor of Clinical Pediatrics, Keck School of Medicine, University of Southern California, Principal Investigator for CAHtalyst Pediatric. "These findings underscore the potential for CRENESSITY to redefine the treatment paradigm for patients with CAH by providing sustained control of androgens and allowing for significant reductions in glucocorticoid dosing – this could ultimately lead to important improvements in key long-term patient outcomes."

Hormone Control and Excess Androgen Outcomes

- Among patients with acne at baseline (visual analog scale [VAS] score >0; range 0-100; mean baseline score: 25.4 mm; n=58), mean acne severity decreased by 6.5 mm at Month 12 (n=52) and by 11.0 mm at Month 24 (n=43), indicating progressive improvement over time.
- In female participants with a baseline hirsutism VAS score >0 (mean baseline score: 27.3 mm; n=29), mean hirsutism scores were largely stable over two years, with a mean change of -6.3 mm at Month 12 (n=27) and +2.4 mm at Month 24 (n=21), despite lower GC doses and continued pubertal stage progression.
- Among male participants Tanner stage 2 or above with an androstenedione-to-testosterone (A4/T) ratio ≥0.5 at baseline (n=32), 31% (9/29) and 36% (9/25) achieved an A4/T ratio <0.5 at Months 12 and 24, respectively.

Supraphysiologic GC Exposure Outcomes

- Among participants with obesity at baseline (body mass index [BMI] ≥85th percentile), 60% achieved clinically meaningful improvements (≥0.2 reduction) in BMI standard deviation score (SDS) at Month 24 with CRENESSITY, including 23% who achieved a BMI <85th percentile.
- Among participants with insulin resistance at baseline, 61% had a homeostatic model assessment of insulin resistance (HOMA-IR) value that no longer met the criteria for insulin resistance with CRENESSITY at two years of treatment.

GC Exposure Outcome Measure	**Baseline**	**Change from Baseline at Month 12**	**Change from Baseline at Month 24**
Mean BMI SDS	1.2 (n=103)	-0.24 (n=97)	-0.30 (n=86)
Percentage of participants who achieved a ≥0.2 reduction in BMI SDS*	— (n=60)	+43% (24/56)	+60% (29/48)
Percentage of participants who achieved a BMI <85th percentile*	— (n=60)	+20% (11/56)	+23% (11/48)
Mean HOMA-IR	3.0 (n=103)	-0.9 (n=87)	-0.6 (n=81)
Percentage of participants who were no longer insulin resistant†	— (n=39)	+57% (20/35)	+61% (20/33)

*Among participants with overweight/obesity (BMI ≥85th percentile) at baseline.
†Among participants with insulin resistance at baseline.

Treatment with CRENESSITY was generally well tolerated by patients, with more than 80% study retention at two years and no new safety signals observed.

Neurocrine recently presented two-year data in adults at the American Association of Clinical Endocrinology 2026 Annual Meeting and plans to share additional two-year data across clinical endpoints and outcomes at upcoming medical meetings.

About Congenital Adrenal Hyperplasia
Congenital adrenal hyperplasia (CAH) is a rare genetic condition that results in an enzyme deficiency that alters the production of adrenal steroid hormones, such as cortisol, aldosterone and adrenal androgens. Severe enzyme deficiency leads to an inability of the adrenal glands to produce enough cortisol and, in approximately 75% of cases, aldosterone. Because individuals with CAH are typically still able to produce androgens, the unused precursors that would normally be used to make cortisol instead result in the production of excess amounts of androgens. If left untreated, CAH can result in adrenal crisis and even death.

Exogenous glucocorticoids (GCs) are necessary to correct the endogenous cortisol deficiency, but historically, doses higher than those needed for cortisol replacement (supraphysiologic) have been used to lower the elevated levels of adrenocorticotropic hormone (ACTH) and adrenal androgens. However, GC treatment at supraphysiologic doses has been associated with serious and significant complications of steroid excess, including metabolic issues such as weight gain and diabetes, cardiovascular disease and osteoporosis. Additionally, long-term treatment with supraphysiologic GCs may have psychological and cognitive impacts, such as changes in mood and memory. Adrenal androgen excess has been associated with abnormal bone growth and development in pediatric patients, female health problems such as excess facial hair growth and menstrual

irregularities, in addition to cardiometabolic and fertility issues in both sexes. The symptoms of high ACTH may include testicular adrenal rest tumors (TARTs).

About CRENESSITY® (crinecerfont)
CRENESSITY is a potent and selective oral corticotropin-releasing factor type 1 receptor (CRF_1) antagonist that reduces and controls excess adrenocorticotropic hormone (ACTH) and adrenal androgens through a non-glucocorticoid (GC) mechanism for the treatment of classic congenital adrenal hyperplasia (CAH). Antagonism of CRF_1 receptors in the pituitary has been shown to decrease ACTH levels, which in turn decreases the production of adrenal androgens and potentially the symptoms associated with CAH. The robust clinical study data demonstrate that lowering adrenal androgen levels with CRENESSITY enables lower, more physiologic dosing of GCs to replace missing cortisol.

CRENESSITY comes in capsules and an oral solution. For adults 18 years of age and older, the recommended dosage is 100 mg twice daily taken orally with a meal. For pediatric patients four to 17 years of age weighing less than 55 kg (121 lbs), the recommended dosage is based on body weight and is administered twice daily, taken orally with a meal. For pediatric patients weighing more than 55 kg (121 lbs), the recommended dosage is 100 mg twice daily taken orally with a meal. Healthcare providers can work with patients to determine the appropriate formulation for use depending on patient needs. Patients receiving CRENESSITY should continue GC therapy for cortisol replacement.

About The CAHtalyst® Studies
The Phase 3 CAHtalyst global registrational studies were designed to evaluate the safety, efficacy and tolerability of CRENESSITY® (crinecerfont) in children and adults with classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency. The CAHtalyst studies were the largest-ever clinical trial program in classic CAH, including 285 pediatric and adult patients.

The CAHtalyst Pediatric study included 103 pediatric patients four to 17 years of age. The study tested two questions. The first question evaluated whether four weeks of CRENESSITY treatment could improve androgen control. The second question evaluated whether an additional 24 weeks of CRENESSITY treatment enabled customized glucocorticoid (GC) down-titration while androstenedione levels were maintained or improved.

The CAHtalyst Adult study included 182 adult patients 18 to 58 years of age. Similarly, the first question of the study evaluated whether four weeks of CRENESSITY treatment could improve androgen control, and the second question evaluated whether an additional 20 weeks of CRENESSITY treatment enabled GC reduction to physiologic range while androstenedione levels were maintained or improved.

Data from the CAHtalyst Phase 3 studies supported approval of CRENESSITY by the U.S. Food and Drug Administration in December 2024. The open-label extension treatment portions of both studies are ongoing.

Important Information

Approved Uses
CRENESSITY® (crinecerfont) is a prescription medicine used together with glucocorticoids (steroids) to control androgen (testosterone-like hormone) levels in adults and children 4 years of age and older with classic congenital adrenal hyperplasia (CAH).

IMPORTANT SAFETY INFORMATION

Do not take CRENESSITY if you:

Are allergic to crinecerfont, or any of the ingredients in CRENESSITY.

CRENESSITY may cause serious side effects, including:

Allergic reactions. Symptoms of an allergic reaction include tightness of the throat, trouble breathing or swallowing, swelling of the lips, tongue, or face, and rash. If you have an allergic reaction to CRENESSITY, get emergency medical help right away and stop taking CRENESSITY.

Risk of Sudden Adrenal Insufficiency or Adrenal Crisis with Too Little Glucocorticoid (Steroid) Medicine. Sudden adrenal insufficiency or adrenal crisis can happen in people with congenital adrenal hyperplasia who are not taking enough glucocorticoid (steroid) medicine. You should continue taking your glucocorticoid (steroid) medicine during treatment with CRENESSITY. Certain conditions such as infection, severe injury, or shock may increase your risk for sudden adrenal insufficiency or adrenal crisis. Tell your healthcare provider if you get a severe injury, infection, illness, or have planned surgery during treatment. Your healthcare provider may need to change your dose of glucocorticoid (steroid) medicine.

Before taking CRENESSITY, tell your healthcare provider about all of your medical conditions, including if you: are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins and herbal supplements.

The most common side effects of CRENESSITY in adults include tiredness, headache, dizziness, joint pain, back pain, decreased appetite, and muscle pain.

The most common side effects of CRENESSITY in children include headache, stomach pain, tiredness, nasal congestion, and nosebleeds.

These are not all the possible side effects of CRENESSITY. Call your healthcare provider for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch at www.fda.gov/medwatch or call 1-800-FDA-1088.

Dosage Forms and Strengths: CRENESSITY is available in 50 mg and 100 mg capsules, and as an oral solution of 50 mg/mL.

Please see full **Prescribing Information**.

About Neurocrine Biosciences, Inc.
Neurocrine Biosciences is a leading biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs. We are dedicated to discovering and developing life-changing treatments for patients with under-addressed neurological, endocrine, psychiatric and immunological disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, chorea associated with Huntington's disease, classic congenital adrenal hyperplasia, endometriosis* and uterine fibroids,* as well as a robust pipeline including multiple compounds in mid- to late-phase clinical development across our core therapeutic areas. For three decades, we have applied our unique insight into neuroscience and the interconnections between brain and body systems to treat complex conditions. We relentlessly pursue medicines to ease the burden of debilitating diseases and disorders because you deserve brave science. For more information, visit neurocrine.com, and follow the company on LinkedIn, X, Facebook and YouTube. *(*in collaboration with AbbVie*)

Forward-Looking Statements
In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements regarding the potential benefits to be derived from CRENESSITY for the treatment of classic congenital adrenal hyperplasia (CAH); the value and benefits CRENESSITY brings to patients with CAH, including its potential to enable patients to transition toward more physiologic glucocorticoid dosing; the ability of Neurocrine Biosciences to ensure patients have access to CRENESSITY; and whether the results from our clinical trials of CRENESSITY are indicative of real-world results. Factors that could cause actual results to differ materially from those stated or implied in the forward-looking statements include, but are not limited to, the following: risks and uncertainties as to whether the data described in this press release will be replicated in additional studies or will be predictive of efficacy or other clinical outcomes in subsequent clinical studies or real-world use of CRENESSITY; risks and uncertainties associated with Neurocrine Biosciences' business and finances in general, as well as risks and uncertainties associated with the commercialization of CRENESSITY, including the extent to which patients and physicians accept and adopt CRENESSITY; whether CRENESSITY receives adequate reimbursement from third-party payors; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY; risks associated with the Company's dependence on third parties for development and manufacturing activities related to CRENESSITY, and the ability of the Company to manage these third parties; risks that additional regulatory submissions for CRENESSITY may not occur or be submitted in a timely manner; risks that the FDA or other regulatory authorities may make adverse decisions regarding CRENESSITY; risks that post-approval CRENESSITY commitments or requirements may be delayed; risks that CRENESSITY may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's annual report on Form 10-K for the year ended December 31, 2025. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof other than required by law.

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Media: Aimee White, 1-858-354-7865, media@neurocrine.com; Investors: Todd Tushla, 1-858-617-7143, ir@neurocrine.com